CLAIMS:

1. An adeno-associated viral vector comprising at least a first polynucleotide that comprises a promoter operably positioned upstream of an isolated nucleic acid segment encoding a biologically-active therapeutic mammalian serpin or cytokine polypeptide, wherein said promoter expresses said nucleic acid segment in a mammalian cell that comprises said vector to produce said encoded mammalian serpin or cytokine polypeptide.

2. The adeno-associated viral vector of claim 1, wherein said nucleic acid segment encodes a therapeutic cytokine polypeptide.

3. The adeno-associated viral vector of claim 2, wherein said polypeptide is selected from the group consisting of α_1-antitrypsin (AAT), a growth factor, an interferon, an anti-apoptosis factor, and a cytokine.

4. The adeno-associated viral vector of claim 3, wherein said polypeptide is selected from the group consisting of BDNF, CNTF, CSF, EGF, FGF, G-SCF, GM-CSF, gonadotropin, IFN, IFG-1, M-CSF, NGF, PDGF, PEDF, TGF, TGF-B2, TNF, VEGF, prolactin, somatotropin, and XIAP1.

5. The adeno-associated viral vector of claim 1, wherein said nucleic acid segment encodes a therapeutic interleukin polypeptide.

6. The adeno-associated viral vector of claim 5, wherein said nucleic acid segment encodes a therapeutic interleukin polypeptide selected from the group consisting of IL-1, IL-2, IL-3, IL-4, IL-5, IL-6, IL-7, IL-8, IL-9, IL-10, viral IL-10, IL-11, IL-12, IL-13, IL-14, IL-15, IL-16, IL-17, and IL-18.

7. The adeno-associated viral vector of claim 1, wherein said promoter is a heterologous, tissue-specific, constitutive or inducible promoter.

8. The adeno-associated viral vector of claim 1, wherein said promoter is a pancreas- or an islet-cell-specific promoter.

9. The adeno-associated viral vector of claim 7, wherein said promoter is selected from the group consisting of a CMV promoter, a β-actin promoter, an insulin promoter, a hybrid CMV promoter, a hybrid β-actin promoter, an EF1 promoter, a U1a promoter, a U1b promoter, a Tet-inducible promoter and a VP16-LexA promoter.

10. The adeno-associated viral vector of claim 9, wherein said promoter is a mammalian β-actin promoter.

11. The adeno-associated viral vector of claim 1, wherein said vector further comprises at least a first enhancer sequence operably linked to said nucleic acid segment.

12. The adeno-associated viral vector of claim 11, wherein said vector further comprises a CMV enhancer, a synthetic enhancer, a liver-specific enhancer, a lung-

specific enhancer, a muscle-specific enhancer, a kidney-specific enhancer, a pancreas-specific enhancer, or an islet cell-specific enhancer.

13. The adeno-associated viral vector of claim 1, wherein said vector further comprises a post-transcriptional regulatory sequence.

14. The adeno-associated viral vector of claim 13, wherein said regulatory sequence comprises a woodchuck hepatitis virus post-transcription regulatory element.

15. The adeno-associated viral vector of claim 1, wherein said mammalian cell is a pancreatic, kidney, muscle epithelial, liver, heart, lung, or brain cell.

16. The adeno-associated viral vector of claim 15, wherein said mammalian cell is a human pancreatic islet cell.

17. A recombinant adeno-associated virus virion comprising the vector of claim 1.

18. The recombinant adeno-associated virus virion of claim 17, wherein said virion is selected from the group consisting of AAV serotype 1, AAV serotype 2, AAV serotype 3, AAV serotype 4, AAV serotype 5, and AAV serotype 6.

19. A plurality of adeno-associated viral particles comprising the vector of claim 1.

20. A mammalian cell comprising the vector of claim 1.

21. The mammalian cell of claim 20, wherein said cell is an endothelial, islet, hepatocyte, pancreas, kidney, muscle, spleen, liver, heart, lung, or brain cell.

23. The mammalian cell of claim 20, wherein said cell is a human cell.

24. A composition comprising the vector of claim 1, the recombinant adeno-associated virus virion of claim 17, the plurality of adeno-associated viral particles of claim 19; or the mammalian cell of claim 20.

25. The composition of claim 24, further comprising a pharmaceutical excipient, buffer, or diluent.

26. The composition of claim 25, formulated for administration to a human.

27. The composition of claim 24, further comprising a liposome, a lipid, a lipid complex, a microsphere, a microparticle, a nanosphere, or a nanoparticle.

28. The composition of claim 24, for use in therapy.

29. The composition of claim 28, for use in cancer, diabetes, autoimmune disease, pancreatic disease, or liver disease therapy.

30. Use of a composition according to claim 24, in the manufacture of a medicament for treating cancer, diabetes, autoimmune disease, pancreatic dysfunction, or liver dysfunction.

31. Use according to claim 30, in the manufacture of a medicament for treating human pancreatic dysfunction.

32. A kit comprising:

(a) the adeno-associated viral vector of claim 1, the virion of claim 17, the viral particles of claim 19, the cell of claim 20, or the composition of claim 24; and

(b) instructions for using said kit.

33. A method for preventing, treating or ameliorating the symptoms of a disease, dysfunction, or deficiency in a mammal, said method comprising administering to said mammal the virion of claim 17, or the viral particles of claim 19 in an amount and for a time sufficient to treat or ameliorate the symptoms of said disease, dysfunction, or deficiency in said mammal.

34. The method of claim 33, wherein said mammal is a human.

35. The method of claim 34, wherein said mammal has, is diagnosed with, or is at risk for developing, diabetes or an autoimmune disorder.

36. The method of claim 33, wherein said virion or said plurality of viral particles is administered to said mammal intramuscularly, intravenously, subcutaneously, intrathecally, intraperitoneally, or by direct injection into an organ or a tissue.

37. The method of claim 36, wherein said organ or tissue is selected from the group consisting of pancreas, liver, heart, lung, brain, kidney, joint, and muscle.

38. A method for treating diabetes in a mammal suspected of having, or at risk for developing diabetes, said method comprising providing to said mammal the composition of claim 24, in an amount and for a time sufficient to treat said diabetes in said mammal.

39. The method of claim 38, wherein said mammal is human.

40. The method of claim 39, wherein said mammal is human with a familial history of diabetes.

41. A method for preventing Type I diabetes in a human suspected of having, or at risk for developing Type I diabetes, said method comprising prophylactically administering to said human the composition of claim 24, in an amount and for a time sufficient to prevent said Type I diabetes from developing in said human.

42. A method for reducing the rate of disease progression of Type I diabetes in a human diagnosed with Type I diabetes, said method comprising administering to said human the composition of claim 24, in an amount and for a time sufficient to reduce the rate of disease progression of said Type I diabetes in said human.